FDA Approves Vemlidy for Children With Hepatitis B

Tenofovir alafenamide is safe and effective for pediatric patients ages 6 and up.

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On March 28, the Food and Drug Administration (FDA) extended the approval of the antiviral medication Vemlidy (tenofovir alafenamide, or TAF) to include children ages 6 and older, according to an announcement from Gilead Sciences. In a Phase II study, half of children who received Vemlidy achieved viral suppression.

Although hepatitis B has declined among children and adolescents since the adoption of universal hepatitis B virus (HBV) vaccination for infants, those who do contract the virus can develop serious liver complications, including cirrhosis and liver cancer.

"Chronic hepatitis B can have a significant and lasting impact on the health of children," Chaun-Hao Lin, MD, of the Keck School of Medicine at the University of Southern California, said in a news release. "As a clinician, I am well aware of the critical importance of promptly treating this disease to avoid possible complications and liver damage. The clinical trial demonstrated that tenofovir alafenamide may represent an effective treatment option for children as young as six years old affected by this chronic disease."

Vemlidy is a nucleoside analog that has demonstrated effectiveness against HBV in numerous studies of adults. It is less likely to cause kidney and bone side effects than the older Viread (tenofovir disoproxil fumarate, or TDF). TAF and TDF are both also widely used for HIV treatment and pre-exposure prophylaxis (PrEP).

The FDA initially approved Vemlidy for adults with chronic hepatitis B. Guidelines from the American Association for the Study of Liver Diseases and the European Association for the Study of the Liver recommend Vemlidy as a preferred treatment for adults. In 2022, the agency expanded the indication to include adolescents ages 12 to 18. Now, it has been extended further to include children down to age 6 who weigh at least 2 kilograms (about 55 pounds). For all age groups, Vemlidy is indicated is for patients with compensated liver disease, meaning the liver is still able to function.

The approval is supported by data from a Phase II clinical trial that included 18 pediatric patients ages 6 to11 who were either receiving hepatitis B treatment for the first time or had previously

tried other antivirals or interferon. Almost all were hepatitis B e antigen (HBeAg) positive. Study participants were randomly assigned to receive Vemlidy or a placebo once daily for 24 weeks; at that point, patients in the placebo group could switch over to open-label Vemlidy.

At 24 weeks, just 8% of children assigned to the Vemlidy group had an undetectable HBV viral load (below 20 IU/mL), compared with none in the placebo group. But this rose to 25% at 48 weeks and 50% at 96 weeks. Although viral suppression rates showed progressive increases over time, they remained below response rates for adults, which are around 60% for HBeAg-positive people and around 90% for HBeAg-negative patients. Children treated with Vemlidy also saw a decline in ALT liver enzyme levels. However, none experienced hepatitis B surface antigen (HBsAg) loss, which is considered a functional cure.

Vemlidy is taken as a pill once daily. It is generally safe and well tolerated. The most common adverse effect is headache. The product label includes a warning that patients who stop taking the antiviral can experience severe hepatitis flare-ups, so liver function should be monitored closely if Vemlidy is discontinued. Unlike direct-acting antiviral therapy for hepatitis C, antivirals for hepatitis B seldom lead to a cure, so treatment is usually ongoing.

Click here for full prescribing information for Vemlidy.
Click here for more news about hepatitis B.
